

TALKING POINTS FOR LARRY KING
March 8, 1994

Why did we pass the Orphan Drug Act --

1980 -- plea from constituent who had Tourette Syndrome

1980/81 -- hearings; numerous rare diseases for which there were potential treatments; drug companies refusing to invest because the drugs not sufficiently profitable

1982 -- passed the Orphan Drug Act over the opposition of drug industry and PMA; to create financial incentives to develop drugs for rare diseases --

- * 7 year monopoly (for the drugs that were not eligible for patents.
- * tax credits for orphan drugs
- * grant program -- \$12 million this year

The Orphan Drug Act has been a phenomenal success.

- * During the decade prior to enactment, less than 10 orphan drugs approved.
- * In the 12 years since enactment, 101 orphan drugs approved. An additional 450 promising compounds have been designated for research.

Important drugs developed under the Orphan Drug Act -- 1993

- * Cystic Fibrosis, which affects 30,000 children
- * Hairy Cell Leukemia
- * Gastaut Syndrome -- a rare seizure disorder in children

Where do we go from here?

- * In a few cases the Act has been used to grant a monopoly for extremely profitable drugs -- drugs that generate \$100s of millions in sales.

Examples: AZT -- HIV

EPO -- Anemia in Kidney dialysis

Human Growth Syndrome -- short kids

Aerosol Pentamidine -- type of pneumonia which is the most common complication of AIDS

Ceredase -- Gaucher's Disease (affects less than 11,000 people)

These drugs cost between \$5,000 and \$25,000 per year. One of them (Ceredase) costs an average of \$150,000 per year.

I have sponsored legislation since 1990 which would change the rules for these extremely profitable drugs -- so that they don't get the full 7-year monopoly, which they don't need in order to generate the capital to stimulate research. Hopefully this legislation can be enacted this year.

An additional issue which we will be looking at this year is the price of drugs developed by the National Institutes of Health. In many cases extremely expensive drugs -- particularly for cancer and AIDS are developed by the National Institutes of Health. My feeling is that where the government has a significant role in developing the drug, it should have a role in determining that the price is not excessive. We are also working on legislation to address this issue as well.